

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

ACHIEVEMENTS REPORT

May 2015

CURING CANCER

Of all the questions addressed to CPRIT,
the toughest is: "When are you going to find a cure for
cancer?" Since cancer is complex and comes in many forms,
its cure will come in diverse forms as well. And with the help
of CPRIT funding, advancements in cancer prevention
and treatment are happening today,
one discovery at a time.

ACHIEVEMENTS AT A GLANCE

CPRIT is a \$3 billion voter-authorized state agency designed to invest in the most promising cancer research, prevention and product development opportunities in the state. As of May 2015, CPRIT has invested \$1.33 billion through more than 900 grants.

(As of May 2015)

CPRIT FUNDING HAS:



CANCER
RESEARCHERS

and their labs to Texas

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EXAMPLE: Herbert Levine, Ph.D.: Recruitment to Rice University from the University of California at San Diego – Dr. Levine is focused on unraveling the complex changes that occur in cells due to cancer, with the aim of finding treatments for currently intractable diseases such as pancreatic cancer.

CREATED AND MAINTAINED MORE THAN

4,700 DIRECT REAL JOBS

Jobs include doctors, scientists, nurses, lab technicians, diagnosticians, health care educators and navigators, data managers, and support personnel.



SUPPORTED NEARLY

CHILDHOOD AND ADOLESCENT cancer research projects

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So far this year, CPRIT has allocated \$17.9 million for academic research projects on childhood and adolescent cancers – a 28 percent increase in funding from 2014.

DELIVERED

2.2 MILLION

PREVENTION SERVICES to Texans

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According to grantee reports, CPRIT's prevention projects have provided over 2 million education and clinical services. Of the 1,105,212 clinical preventive services provided, there have been 528,645 screenings and diagnostics for breast, cervical and colorectal cancer. Of these:

- 42,991 abnormal results were identified;
- 3,340 cancer precursors were detected, and
- 1,477 cancers were detected.

Every \$1 spent through CPRIT for screening/prevention leads to \$7.16 in treatment cost savings, preserved productivity, and other economic benefits through earlier detection of cancers.

PRODUCT DEVELOPMENT GRANT CONNECTIONS to Texas academic institutions

EXAMPLE: AERase, Inc., based at the UT Austin Incubator Center, is working on a cancer therapy that uses human enzymes to starve and kill cancer cells.



EXAMPLE: Dr. Havinder Gill, Texas Tech
University, "Coating solid dispersions on
microneedles via a molten dip-coating
method," a device to treat difficult to reach
tumors of the tongue. Published in the *Journal*of Pharmaceutical Sciences. (Sep. 2014)

RESULTED IN

84 NEW CLINICAL TRIALS

(5.593 PATIENTS) Click here to read more...

EXAMPLE: Hak Choy, Technology-directed advances in radiation therapy of lung cancer – New technologies in radiation therapy are being tested, which may allow higher doses of radiation to be delivered in fewer treatments with fewer side effects.

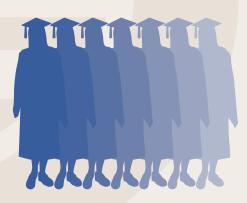


\$910.8 MILLION in follow-on investment in CPRIT-supported companies

- \$197 million in state tax receipts and \$92.1 million in local tax receipts can be attributed to CPRIT's activities and investments in 2014.
 (The Perryman Group report)
- CPRIT funding has supported more than 20 companies across Texas.

ENABLED TRAINING OF MORE THAN

1,000 COLLEGE AND GRADUATE LEVEL STUDENTS



EXAMPLE: Baylor College of Medicine, Translational Biology and Molecular Medicine Training Program – Graduate student Tonia Kurtova worked on a research team that discovered why bladder cancer cells resist chemotherapy treatment. She is the lead author of a report on the study published in the prestigious journal *Nature*.

Visit www.cprit.state.tx.us/news/achievements for updates and additional information on the CPRIT achievements.

PROGRAM HIGHLIGHTS

Here are six examples of CPRIT's grantees achieving real results – one discovery at a time.

ACADEMIC RESEARCH PROGRAM

The goal of CPRIT's research program is to discover new information about cancer that can lead to prevention, early detection, and more effective treatments; translate new and existing discoveries into practice for cancer diagnosis, treatment, and survivorship; and increase the prominence and stature of Texas in the fight against cancer. As of May 2015:

- Total Number of Research Grants Awarded: 735
- Total Amount of Research Grants Awarded: \$928,542,585
- Total Number of Researcher Recruitment Grants Awarded: 88
- Total Amount of Recruitment Grants Awarded: \$253,459,916

CPRIT Grants are Enhancing Research Superiority in Texas

Grantee: Dr. Zeina Nahleh, Texas Tech University Health Science Center in El Paso

Project: Establishing a Cancer Clinical Research Core facility at
Texas Tech University Health Science Center in El Paso.
This center allows activation of currently funded cancerrelated projects, development of an educational and
community outreach office to expand patient awareness
of cancer programs, and of a new cancer database for
sharing key data with Texas clinical oncology clinical trials

Impact: Clinical trials are now available to the Latino community in the El Paso area.

Grantee: Dr. James Allison, The University of Texas M.D. Anderson Center

Project: CPRIT Scholar: Recruitment of Established Investigator.

With the help of a CPRIT recruitment grant, M.D. Anderson brought noted researcher Jim Allison, Ph.D. to Texas.

Allison's research has exposed ways to use the immune system to attack cancers, revealing an entirely new way to combat these diseases. This groundbreaking discovery earned him the 2014 Breakthrough Prize in Life Sciences among many other distinguished recognitions.

Impact: Dr. Allison's work has opened a new field of immunotherapy, improved the survival of patients with advanced melanoma and is now being extended to other types of cancer.

PRODUCT DEVELOPMENT RESEARCH PROGRAM

CPRIT's product development program seeks to identify private sector entities to develop products that will benefit cancer patients. As of May 2015:

- Total Number of Product Development Grants Awarded: 27
- Total Amount of Product Development Grants Awarded: \$258.540.542
- Total Investment in Research and Development (with matching funds): \$281,877,121
- · Jobs Created by Product Development Projects: approximately 211

CPRIT Grants are Moving Science into the Clinic, Supporting New Therapies

Grantee: CerRx, Inc.

Project: Novel Ceramide-Modulating Therapeutics for Cancer Use.

Developing drugs that trick cancer cells into overproducing toxic waxes, called ceramides, which cells need to grow and divide. When the ceramides increase to a certain level, the cancer cells die. CerRx has a pipeline of such drugs, which work synergistically against many cancer types in laboratory testing.

Impact: Clinical testing has already shown that one of CerRx's drugs can eliminate the cancers of some patients with relapsed lymphomas. CerRx uses its CPRIT funding to advance these drugs to market by conducting additional clinical trials in Texas.

Grantee: DNAtrix

Project: Clinical Development and Commercialization of Oncolytic
Adenovirus for Treating Malignant Glioma.

Modified the common cold virus in ways to develop a
virus-based therapy for the treatment of one of the most
aggressive types of brain cancer.

Impact: In human trials, Delta-24-RGC showed a remarkable ability to hunt and kill tumors and to improve the survival rates of patients. If additional clinical trials prove successful, the FDA is expected to approve the product.

PREVENTION PROGRAM

Through the prevention program, CPRIT supports the provision of state-of-the-art preventive clinical services and tailored, culturally appropriate information to the public and health professionals with a focus on providing access to populations in most need and those disproportionately affected by cancer. As of May 2015:

- Total Number of Prevention Grants Awarded: 146
- Total Amount of Prevention Grants Awarded: \$142,189,920
- Total Number of Prevention Services Delivered to Texans:
 2.2 million

CPRIT Grants are Reaching Underserved Populations, Saving Lives

Grantee: The University of Texas Southwestern Medical Center

Project: Telemedicine Genetic Counseling.

Through the only telemedicine program of its kind in Texas, genetic counseling is provided to populations who have never received services with the goal of identifying patients with Hereditary Breast-Ovarian Cancer (HBOC) and Lynch syndrome, two of the most commonly inherited cancer predisposition syndromes.

Impact: In the first 20 months, more than 61,000 underserved women were screened for HBOC risk and more than 500 underserved patients with colon and uterine cancer were tested for Lynch syndrome. Additional funding from CPRIT will expand the reach of this project from six to 22 counties in North Texas.

Grantee: Dr. Carol Rice, Texas A&M AgriLife Extension Service

Project: Targets underserved women in 50 counties to help them get breast and cervical cancer screenings.

Impact: Screenings of 3,157 women have detected 198 abnormalities, including 42 cancer pre-cursors. Indicating the high demand for services, the project has reached 7,590 women in nearly three years—more than twice the goal.



CURING CANCER: WHERE ARE WE TODAY?

A Q&A with Dr. Margaret Kripke, CPRIT Chief Scientific Officer

1. HOW LIKELY IS IT THAT WE WILL BE ABLE TO CURE CANCER IN THE NEAR FUTURE?

Actually, cancers are being cured every day. Around two-thirds of people who develop cancer remain cancer-free for five or more years, more than half are cured of their disease, and the number of cancer survivors grows daily. For people who are not cured by today's treatments, we continue to investigate new approaches. It is unlikely a single breakthrough will lead to the cure of all cancers. That is because there are many different types of cancer that require different treatments. Not only are cancers of the lung different from breast cancers, but there are many different subtypes of lung and breast cancers, each of which may require a different treatment. Although we are making steady gains in providing new, less toxic treatments for cancer, it will be a long time before we have an effective therapy for every cancer type.

2. Why is cancer so difficult to cure?

In addition to the fact that there are many different subtypes of cancer, each of which may require a different treatment, cancer cells have two characteristics that make them a challenge to treat. One is their ability to spread to other sites of the body (metastasis). This means that removing the original cancer by surgery or radiation will not necessarily cure the disease, which can appear elsewhere in the body months or years after removal of the primary cancer. Thus, some type of systemic therapy is generally required to eliminate cancer cells that have already spread at the time of diagnosis. The second characteristic is the ability of cancer cells to develop resistance to chemotherapy and radiation. Most cancers have the ability to change their properties so that they become resistant to treatment. Just like the common cold virus or the influenza virus, cancer cells can change, or mutate, to become resistant to therapy. When one treatment becomes ineffective, another one is needed, but in many cases, no other options are available.

3. What are the most promising avenues of cancer research today?

Research in recent years has revealed that cancer cells have changes in their DNA that distinguish them from normal cells. Some of these changes enable the cells to escape the normal processes that control cell division and cell death. Finding new drugs that target these changes is an intense area of investigation. Such drugs would act against the cancer cells, but would not harm normal cells, thus sparing patients the toxicity that accompanies current chemotherapy. An example of such targeted therapy is gleevec, which destroys cancer cells having a particular mutation in their DNA. Many more such drugs are under development. Another promising avenue is early detection. Cancers that are found early are more easily cured than advanced cancers that have already spread to distant organs. Many current efforts are directed to development of new screening tests for cancer, particularly pancreatic and ovarian cancers, which are often diagnosed late and are therefore very difficult to cure. Another important focus is using the body's immune system to combat cancer. New findings regarding how the immune system is controlled are being applied to the treatment of melanoma skin cancers with considerable success. Current research is attempting to extend these findings to other types of cancer.

4. WHAT WOULD MAKE THE BIGGEST DIFFERENCE IN REDUCING THE BURDEN OF CANCER?

Eliminating tobacco use would have the most dramatic effect on reducing cancer incidence and cancer deaths. In the United States alone, deaths from lung cancer would be reduced by 80 percent, and deaths from all cancers would be reduced by 30 percent within 20 years after people stopped using tobacco. Other approaches to preventing cancer, such as exercise, better eating habits, and removing toxic chemicals from the environment would also reduce the incidence of cancer over time. Preventive measures have the added benefit of sparing people from the emotional, physical, and financial toll resulting from a cancer diagnosis. After prevention, the

next biggest difference would result from early detection of cancer, for the reasons stated above. For cancer treatment, understanding the mechanisms by which cancer cells become resistant to therapy and finding ways to counteract them is critical to ensuring the effectiveness of all forms of treatment. Finally, improving the access of underserved populations to today's standard of cancer care would also reduce the burden of cancer and would help to address the large disparities in outcomes of cancer treatment that result from differences in socio-economic status.

5. How is CPRIT addressing these challenges?

CPRIT's prevention program is bringing cancer risk reduction, screening and survivor programs to rural and underserved populations across the state. These efforts help people reduce the risk of getting cancer, identify cancer earlier and assist them in finding appropriate cancer treatment options, thus reducing the overall burden of cancer in the state. The research program is helping research institutions to recruit the most talented cancer researchers to Texas. It also supports cutting-edge research projects that range from understanding how cancer cells are formed, spread, and develop resistance, to development of new anti-cancer drugs and new tools for cancer diagnosis and imaging. The product development program is moving advances in research out of the laboratory and into the development of devices, drugs, and biological agents for use in cancer detection and treatment.





ATTRACTED NEARLY

CANCER RESEARCHERS and their labs to Texas.

Building Research Strength: CPRIT funding has attracted nearly 90 cancer researchers and their labs to Texas. (Nov. 2014)

Ralf Kittler, Ph.D., Recruitment to UT Southwestern Medical Center from the University of Chicago. Nationally renowned for his work on the biology of cancers, Dr. Kittler's objectives include the development of new methods for detection and treatment of prostate cancer.

Herbert Levine, Ph.D., Recruitment to Rice University from the University of California at San Diego. Dr. Levine is focused on unraveling the complex changes that occur in cells due to cancer, with the aim of finding treatments for currently intractable diseases such as pancreatic cancer.

Li Ma, Ph.D., Recruitment to the University of Texas MD Anderson Cancer Center from MIT's Whitehead Institute for Biomedical Research. Dr. Ma is investigating the roles and mechanisms of small RNA molecules in regulating tumor metastasis, and will develop new candidate therapies for cancer.

DELIVERED OVER

2 MILLION

PREVENTION SERVICES TO TEXANS.



Saving Lives: CPRIT funding has provided prevention services in all 254 Texas counties. (May 2015)

More than one million education and training services, and 1,105,212 clinical services have been delivered to Texans. According to grantee reports, these projects in the CPRIT prevention portfolio have led to:

16,562 prevention vaccinations

17,036 genetic testing and counseling services

189,842 tobacco cessation services

10,743 survivor care services

528,645

screenings and diagnostics for breast, cervical and colorectal cancer. Of these:

- 212,936 recipients had never before been screened;
- 42,991 abnormal results were identified;
- 3,340 cancer precursors were detected; and
- 1,477 cancers were detected.



The work of CPRIT grantees has appeared in such prestigious publications as the journal Nature. (Jan. 2015)

Cancer, Epidemiology, Biomarkers & Prevention (Nov. 2014)

"Colorectal cancer screening for African Americans: Should we be screening at an earlier age?" by Dr. Samir Gupta (The University of Texas Southwestern Medical Center)

Proceedings of the National Academy of Sciences (Jan. 2015)

"Jagged Delta asymmetry in Notch signaling can give rise to a Sender/Receiver hybrid phenotype" by Dr. Jose Onuchic (Rice University)

Blood (American Society of Hematology journal) (Jan. 2014)

"Experimental gene therapy successful in certain lymphomas and leukemia" by Dr. Laurence Cooper (The University of Texas MD Anderson Cancer Center)

Journal of Pharmaceutical Sciences (Sept. 2014)

"Coating solid dispersions on microneedles via a molten dip-coating method," a device to treat difficult to reach tumors of the tongue, by Dr. Havinder Gill (Texas Tech University)

Nature (Jan. 2015)

Targeting the "wound response" of cancer stem cells may explain why bladder cancer stem cells are so resistant to chemotherapy by Antonina Kurtova (Graduate student, Baylor College of Medicine)

RESULTED IN

84 NEW CLINICAL TRIALS

Helping more people: CPRIT funding has resulted in the enrollment of 5,593 patients in 84 new clinical trials. (Jan. 2015)

Hak Choy (UT Southwestern), *Technology-directed advances in radiation therapy of lung cancer*. New technologies in radiation therapy are being tried, which may allow higher doses of radiation to be delivered in fewer treatments with fewer side effects.

Louis Chrystal (Baylor College of Medicine), Chemo-immunotherapy for relapsed or refractory nasopharyngeal carcinoma (NPC). Chemotherapy is used to reduce the size of tumors, followed by immunotherapy to remove remaining cells in cancers induced by the Epstein-Barr virus.

Carlos Beccera (Baylor College of Medicine), Novel dendritic cell vaccine therapy for pancreatic cancer. Patients with pancreatic cancer are being immunized against their cancer using dendritic cells – cells of the immune system that trigger an immune response. The study involves improving the effectiveness of these dendritic cell vaccines.

Michael Davies (UT MD Anderson Cancer Center), Clinical and molecular characteristics of combined BRAF and MEK inhibitor treatment in patients with metastatic melanoma resistant to BRAF inhibitor alone. A growth blocker against melanoma has proven to work only temporarily, and resistant cells eventually develop. Patients in this trial are being treated with the growth blocker and one other that targets the resistant cells to see if the combination improves patients' survival.

Laurence Cooper (UT MD Anderson Cancer Center), Clinical application of umbilical cord blood-derived designer T cells. Tumors in patients with aggressive leukemias and lymphomas often relapse following treatment with transplants of umbilical cord blood lymphocytes. In this clinical trial, genetically modified T-cells are engineered to attack a specific molecule on the leukemia cells to enhance treatment.